Human Stem Cell Approaches to Alzheimers Disease Therapies

Grant Award Details

Human Stem Cell Approaches to Alzheimers Disease Therapies

Grant Type: Disease Team Planning

Grant Number: DT1-00675

Investigator:

Name:	Lawrence Goldstein
Institution:	University of California, San Diego
Type:	PI

Award Value: $55,000

Status: Closed

Grant Application Details

Application Title: Human Stem Cell Approaches to Alzheimers Disease Therapies

Public Abstract: Alzheimer's Disease (AD) is a major health problem in the U.S. with 10% of people over the age of 65 and 50% of people over the age of 85 afflicted with disease. In spite of the magnitude of the problem, however, we lack a compelling mechanistic understanding of AD. As a result, we have no effective therapeutic agents for AD, with few if any on the horizon. While cellular therapy might some day be a viable therapy for AD, current data do not strongly support such an approach and the obstacles to implementing cellular therapy for AD include the lack of a compelling mechanism of neurodegeneration. We propose to recruit a disease team that will use human stem cells to accelerate the development and prioritization of drug-like therapeutic agents as well as developing targets for therapeutic agent development based on the mechanisms identified. Importantly, the resulting cellular and animal models can also be used as assays to screen chemical libraries to find novel drugs that may protect against the degenerative processes. These investigations will require significant interdisciplinary activity as they will need technologies, informatics, and computing beyond those typical of most stem cell biology laboratories. Our general plan is to use funds obtained for workshops, bringing in outside experts to consult, to define a pathway and set of processes and assays to test AD therapeutics, begin developing a disease team application, and to identify one or more private sector collaborators, who will commit to collaborating. Finally, although therapy development for AD might not be as conceptually mature as for some other diseases, the significance and social and financial impact of AD is massive. In addition, since clinical trials for AD are already proceeding in large number, the potential ability to design and use human cellular models to improve the therapy development and prioritization process is very likely to have a major impact on what agents enter, and hopefully succeed, in clinical trials in a 4-5 year period.

Statement of Benefit to California:	Alzheimer's Disease (AD) is a major health problem in the U.S. with 10% of people over the age of 65 and 50% of people over the age of 85 afflicted with disease. In spite of the magnitude of the problem, however, we lack a compelling mechanistic understanding of AD. As a result, we have no effective therapeutic agents for AD, with few if any on the horizon. While cellular therapy might some day be a viable therapy for AD, current data do not strongly support such an approach and the obstacles to implementing cellular therapy for AD include the lack of a compelling mechanism of neurodegeneration. We propose to recruit a disease team that will use human stem cells to accelerate the development and prioritization of drug-like therapeutic agents as well as developing targets for therapeutic agent development based on the mechanisms identified. Because AD is such an expensive disease with major social and familial impact, progress in therapeutic development will not only strengthen California businesses, but may also provide some relief from the enormous costs of caring for people with AD.

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